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## **Pharmacyclics® Announces Updated Results for BTK Inhibitor Ibrutinib (PCI-32765) at American Society of Clinical Oncology (ASCO) Annual Meeting**

### **Investigational agent ibrutinib (PCI-32765) demonstrates progression-free survival (PFS) in treatment naive patients and high response rates with combinations in relapsed/refractory patients with advanced CLL/SLL**

CHICAGO, June 4, 2012 /PRNewswire/ -- Pharmacyclics, Inc (Nasdaq: PCYC) announced today updated results from three trials of Bruton's tyrosine kinase (BTK) inhibitor, ibrutinib (PCI-32765), for the treatment of patients with chronic lymphocytic leukemia (CLL)/small lymphocytic lymphoma (SLL).

"This meeting at ASCO has been a continued affirmation of the promising activity and clinical benefit of ibrutinib in CLL. It will be exciting to move this toward definitive Phase III study to get this drug approved for CLL patients," said John Byrd, M.D., D. Warren Brown Chair of Leukemia Research and Director, Division of Hematology at Ohio State University Comprehensive Cancer Center — Arthur G. James Cancer Hospital and Richard J. Solove Research Institute (OSUCCC — James).

The ibrutinib clinical updates at ASCO released in two oral presentations and a poster discussion session include: 1) safety and efficacy data from the Phase Ib/II CLL/SLL single agent trial in treatment naive patients (PCYC-1102); 2) safety and efficacy data from the Phase Ib/II CLL/SLL combination trial with ofatumumab in relapsed or refractory patients (PCYC-1109); and 3) safety and efficacy data from the Phase Ib/II CLL/SLL combination trial with bendamustine and rituximab in relapsed or refractory patients (PCYC-1108).

#### **Abstract # 6507: The Bruton's tyrosine kinase inhibitor PCI-32765 in treatment-naïve chronic lymphocytic leukemia patients: Interim results of a phase Ib/II study.**

Dr. John C. Byrd et al., The Ohio State University, Columbus, Ohio.

- Non-hematologic toxicities of ibrutinib single agent remain manageable and tolerable with no new signals; hematologic toxicities were uncommon.
- Overall response rate in the 420 mg cohort is 81% using ibrutinib as a single agent. 12% of patients achieved a complete response with no morphologic evidence of CLL. Progression free survival with a median follow-up of 14.4 months is 96% in the 420 mg cohort.

This trial (PCYC-1102) included a total of 31 patients with CLL/SLL enrolled at two fixed continuous dose levels of ibrutinib single agent, 420 mg (n=26) and 840 mg (n=5), respectively. With a median follow-up of 14.4 months in the 420 mg cohort, the overall response rate, including complete and partial responses, was 81% as measured by the 2008 International Workshop on Chronic Lymphocytic Leukemia (IWCLL) criteria. The clinical responses have been independent of high-risk clinical or genetic features. Progression free survival with 14.4 months median follow-up is 96% in the 420 mg cohort. The study safety profile of ibrutinib was particularly notable for minimal off target toxicities and was consistent to earlier trials with most common adverse events Grade 1/2 diarrhea, nausea, and fatigue. Grade 3 and Grade 4 hematologic events potentially related to ibrutinib were 12%. Of the 31 patients on the trial, there has only been 1 patient that has discontinued due to disease progression. Overall, these data support Phase III evaluation of ibrutinib as a single agent in treatment naïve CLL/SLL patients. This abstract was selected to be presented at the "Best of ASCO Meetings."

#### **Abstract # 6508: A phase Ib/II study evaluating activity and tolerability of BTK inhibitor PCI-32765 and ofatumumab in patients with chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) and related diseases.**

Dr. Samantha Mary Jaglowski et al. The Ohio State University, Columbus, Ohio.

- Most adverse events were Grade 1 and 2, and commonly reported Grade 3 infectious events were as expected in this patient population
- Overall response rate is 100% in CLL/SLL/PLL patients. Progression free survival with a median follow-up of 9.8 months is 100%.

The combination trial (PCYC-1109) included a total of 27 patients with CLL/SLL/PLL (n=24) and Richter's transformation (n=3) that were treated in cohort one, in which ibrutinib (420 mg) was followed by concomitant ofatumumab with continued ibrutinib until progression. The combination was well tolerated with the majority of adverse events Grades 1/2. No new safety signals were identified. For the CLL/SLL/PLL patients the overall response rate, as measured by IWCLL criteria, and progression free survival is 100% with a median follow-up of 9.8 months. 89% of CLL/SLL/PLL patients remain on study and only 1 patient has discontinued treatment by proceeding to stem cell transplant. Achievement of 100% tumor response, rapid onset of response, low relapse rate with durable responses, and a favorable safety profile make this combination worthy of further study. Cohorts evaluating other therapeutic sequences are currently underway.

**Abstract # 6515: Combination of the Bruton's tyrosine kinase inhibitor PCI-32765 with bendamustine/rituximab (BR) in patients with relapsed/refractory chronic lymphocytic leukemia: Interim results of a phase Ib/II study.**

Dr. Susan M. O'Brien et al. MD Anderson Cancer Center, Houston, Texas.

- Adverse events are consistent with previous reports of the BR combination. No new safety signals with the combination of ibrutinib and BR were identified.
- Overall response rate is 93%, with 13% of patients achieving a complete response with no morphologic evidence of CLL.

This combination trial (PCYC-1108) enrolled a total of 30 patients; 37% were considered refractory (treatment free interval < 12 mo) to a purine analog containing regimen and 13% refractory to bendamustine. There have been no discontinuations due to adverse events. With a median follow up of 8.1 months only 2 patients have reported progressive disease and an additional 5 patients have proceeded to stem cell transplant, 23 (77%) of patients remain on study. The high overall response rate, low rate of progressive disease, and good tolerability compares favorably with historical controls, warranting a randomized Phase 3 study of ibrutinib in combination with bendamustine/rituximab.

"In my opinion the Pharmacyclics ibrutinib data released at ASCO is phenomenal. The data support our stated mission 'to improve the quality and duration of life for patients suffering from oncologic diseases'," said Bob Duggan, CEO and Chairman of the Board of Directors.

**Conference Call Details**

The Company will be holding a conference call on Wednesday, June 6, 2012 at 4.30pm ET. To participate in the conference call, please dial 1-877-407-0778 for domestic callers and 1-201-689-8565 for international callers. To access the live audio broadcast or the subsequent archived recording, log on to <http://ir.pharmacyclics.com/events.cfm>. The archived version of the webcast will be available for 30 days on the Investor Relations section of the company's Web site at [www.pharmacyclics.com](http://www.pharmacyclics.com).

**About Pharmacyclics**

Pharmacyclics® is a clinical-stage biopharmaceutical company focused on developing and commercializing innovative small-molecule drugs for the treatment of cancer and immune mediated diseases. Our mission and goal is to build a viable biopharmaceutical company that designs, develops and commercializes novel therapies intended to improve quality of life, increase duration of life and resolve serious unmet medical healthcare needs; and to identify promising product candidates based on scientific development expertise, develop our products in a rapid, cost-efficient manner and pursue commercialization and/or development partners when and where appropriate.

Presently, Pharmacyclics has three product candidates in clinical development and several preclinical molecules in lead optimization. We are committed to high standards of ethics, scientific rigor, and operational efficiency as we move each of these programs to viable commercialization.

The Company is headquartered in Sunnyvale, California and is listed on NASDAQ under the symbol PCYC. To learn more about how Pharmacyclics advances science to improve human healthcare visit us at <http://www.pharmacyclics.com>.

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